U.S. Food and Drug Administration

SINGLE PATIENT EXPANDED ACCESS:

WHAT YOU NEED TO KNOW

Q. What is single patient expanded access, and do I qualify?

A. Expanded access is the use of an investigational drug outside of clinical trials to diagnose, monitor, or treat serious or life-threatening illnesses. Your physician will help guide you through the process.

To obtain expanded access, you must:

- Have a serious or immediately life-threatening disease or condition;
- Have no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; and
- Generally be unable to participate in a clinical trial.

Q. What are the risks to me?

A. Because these are investigational drugs and they are not yet FDA-approved, there may be unknown risks.

Q. What helps protect me from risk?

A. Your doctor must request approval for expanded access use of a drug from an Institutional Review Board (IRB) and authorization from FDA. An IRB is a committee that reviews the plan for expanded access use to assure that your rights and welfare are protected. Before starting treatment, your doctor must give you an informed consent document to sign to make sure you understand the potential risks associated with the drug. FDA will only authorize the application if your doctor makes a determination that the risk of using an experimental medicine are justified in your case.

For more information about drugs in development and clinical trials around the world, visit www.clinicaltrials.gov.
You can also contact the drug company or patient advocacy organizations to see if they have information on expanded access programs or ongoing clinical trials.

Q. How do I request expanded access?

A. Physicians request expanded access from the FDA for their patients. The physician should first contact the drug company to make sure it is willing to provide an investigational drug to you before requesting expanded access from the FDA. FDA cannot make a drug company provide their investigational drug for expanded access; they must do so voluntarily. The approval from the drug company is a letter of authorization (LOA).

Q. If I am authorized for expanded access, when can I begin treatment?

A. If the drug company agrees to provide the drug, you may begin treatment 30 days after FDA receives the request, unless your doctor receives earlier notification from FDA that the treatment may proceed. Your doctor must also receive IRB approval before treatment can begin. FDA reviews and responds to expanded access requests often within days of receiving the request. Your physician is responsible for administering the drug and monitoring you carefully.

Q. What are the procedures if I need the drug on an emergency basis?

A. In an emergency, FDA can grant access over the phone and treatment can start once your physician receives the medication from the drug company. However, your physician must complete an expanded access application within 15 working days and notify an IRB within 5 days of obtaining approval from FDA.

Q. How much will it cost? Will insurance cover it?

A. The treating physician may request authorization from FDA to charge for an investigational drug and can only charge you the direct costs of making the drug available, such as manufacturing and shipping. Any additional costs for administering the drug and monitoring its use will depend on your insurance coverage and do not require FDA authorization. FDA has no authority to require that the Centers for Medicare and Medicaid Services (CMS) or any private health insurance company reimburse for investigational drugs for which FDA has authorized charging. It is important that you and your physician consider the cost of the investigational drug and the medical services associated with its use that are not covered by third-party payers such as insurance or Medicare.

Q. What are some reasons I might not receive expanded access?

A. The drug company may choose not to provide the drug or there may not be enough drug supply. The FDA may deny a request for a number of reasons, including if there are available clinical trials for that drug or if the clinical severity of the disease or condition does not outweigh the risks of the drug. *However, FDA grants expanded access for almost all of the applications it receives.*

Contact PatientNetwork@fda.hhs.gov or 301-796-8460 with any questions.

More Information:

- FDA Information for Patients: Expanded Access
- FDA Guidance: Expanded Access to Investigational Drugs for Treatment Use - Questions and Answers
- Application for Individual Patient Expanded Access

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